Stem Cell Therapy for Muscular Dystrophy

Grant Award Details

Stem Cell Therapy for Muscular Dystrophy

Grant Type: Disease Team Planning

Grant Number: DT1-00674

Investigator:

Name:	Thomas Rando
Institution:	Stanford University
Type:	PI

Award Value: $0

Status: Closed

Grant Application Details

Application Title: Stem Cell Therapy for Muscular Dystrophy

Public Abstract:

The goal of our team is to capitalize on the remarkable advances in stem cell biology to apply those advances to the treatment of human diseases, disorders, and injuries. We are planning to develop and initiate a clinical trial that would use stem cells for the treatment of muscular dystrophies. This application of stem cell therapy has the potential to benefit thousand of individuals, adults and children alike, who have one of the many different types of muscular dystrophy. Moreover, the success of this clinical trial would open the door for the possible use of stem cells in a much wider range of muscle disorders that afflict our population including the treatment of muscle wasting that accompanies prolonged bedrest, many chronic diseases such as AIDS and cancer, and even the muscle loss that accompanies normal aging. Additionally, because we have the advantage of a unique opportunity to study stem cell therapeutics in these well-defined muscular dystrophies, the technological advances that will accompany these studies will likely be applicable to the use of stem cells for the treatment of diverse chronic and degenerative conditions of other solid tissues such as the heart, the liver, and the brain.

Among our core team members, there is expertise that includes the basic biology of muscle stem cells and human embryonic stem cells, the use of stem cells in the treatment of muscular dystrophies, and the clinical diagnosis and treatment of humans with muscular dystrophies. We have two main goals of this six-month planning phase. First, we will focus our individual and collaborative efforts on research directions that will be targeted to this specific therapeutic application. This will involve the establishment of specific milestones that we will need to reach in that short time. Second, we will hold regular meetings to consider challenges that we are likely to face in the years ahead, and the solutions will be to expand our team with collaborators and consultants who will be important to the success of our program. These individuals will be included as associate members of the team and will participate in subsequent planning sessions. Additionally, we will form a core Advisory Board consisting of individuals from academia, industry, regulatory agencies, and the general public. This will insure a diverse source of advice and guidance with regard to solutions to problems and anticipation of hurdles that we will need to address. Finally, during this six-month planning phase, we will develop a full proposal that will be ready for submission to CIRM to apply for funding for this major program in stem cell therapeutics. At the conclusion of the six-month planning phase, we will have increased the momentum of our program for this important area of stem cell therapeutics. Our overall goals, specific objectives and proposed program meet the spirit and the letter of CIRM's mission and that fulfill the promise that CIRM holds for the citizens of California.

Statement of Benefit to California:	The specific benefit that the success of this planning grant will have for the citizens of the State of California will be realized in the successful planning and execution of the subsequent clinical trial of stem cells in the treatment of muscular dystrophies. Our assembled team has the expertise to capitalize on a unique opportunity at this moment in which advances in stem cell biology, the initiation of numerous clinical trials for muscular dystrophies, and advances in diagnostics methodologies have resulted in a tremendous momentum for translating decades of research into effective clinical treatments for this devastating group of diseases. The success of a stem cell therapeutic approach would represent a true milestone in the treatment of the most common form of muscular dystrophy, Duchenne muscular dystrophy, which is the most common lethal, hereditary disease of childhood. In addition to the historic watershed, the success of such a trial would open the way for the use of stem cells in the treatment of other forms of muscular dystrophy that lead to disability and premature death in adults and children alike. It would also expand the scope of muscle diseases, disorders, and injuries that could potentially be treatable by stem cells. Such conditions would include the profound muscle wasting that accompanies chronic diseases such as AIDS and cancer, as well as the muscle loss that accompanies normal aging. As such, there is great potential for the alleviation of individual suffering and restoration of function for tens of thousands of California citizens and millions of people worldwide. Such success would also carry an enormous economic benefit in terms of elimination of medical costs for the long-term care of individuals, as well as the benefit of increased productivity. Beyond the obvious individual and collective health and economic benefits associated with treatment of any chronic disease, the success of a clinical trial of stem cells in the treatment of muscular dystrophies would have additional benefits in terms of stem cell therapeutics and regenerative medicine. In the process of designing, conducting, and analyzing the results of the clinical trial, we will certainly pave the way for further trials of stem cells for the treatment of diseases and injuries of solid organs such as heart and brain. Many of the technologies we will develop, including optimization of conditions for growth of stem cells, methodologies for delivery of stem cells to tissues, and technologies for imaging the cells within the body once they are there, are certain to be of value for stem cell therapeutics across a wide range of organs and diseases. This will further propel the CIRM mission and lead to further benefits for the citizens of the State of California, on top of which is the potential benefit of the growth an important, new biotechnology industry to partner with academic centers and government programs for the advancement of regenerative medicine.

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